4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2015-D-2818]

Rare Diseases: Common Issues in Drug Development; Draft Guidance for Industry; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a draft guidance for industry entitled "Rare Diseases: Common Issues in Drug Development." The purpose of this draft guidance is to advance and facilitate the development of drugs and biologics to treat rare diseases. Drug development for rare diseases has many challenges related to the nature of these diseases. This draft guidance is intended to assist sponsors of drug and biological products for treating rare diseases in conducting more efficient and successful development programs.

DATES: Although you can comment on any guidance at any time (see 21 CFR 10.115(g)(5)), to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance, submit either electronic or written comments on the draft guidance by [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE FEDERAL REGISTER].

ADDRESSES: Submit written requests for single copies of the draft guidance to the Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration, 10001 New Hampshire Ave., Hillandale Building, 4th Floor, Silver Spring, MD 20993-0002; or Office of Communication, Outreach, and Development, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm. 3128, Silver Spring, MD 20993-0002. Send one self-addressed adhesive label to assist that office in processing your requests. See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

Submit electronic comments on the draft guidance to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Jonathan Goldsmith, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, rm 6480, Silver Spring, MD 20903-0002, 240-402-9959; or Stephen Ripley, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance for industry entitled “Rare Diseases: Common Issues in Drug Development.” This guidance is intended to assist sponsors of drug and biological products for treating rare diseases in conducting more efficient and

successful development programs through a discussion of selected issues commonly encountered in rare disease drug development. Although these issues are encountered in other drug development programs, they are frequently more difficult to address in the context of a rare disease than a common disease for which there is greater and more widespread medical experience. These issues are also more acute with increasing rarity of the disorder. A rare disease is defined by the Orphan Drug Act as a disorder or condition that affects less than 200,000 persons in the United States; however, most rare diseases affect far fewer persons.

Most rare disorders are serious conditions with no approved treatments, and rare disease patients have considerable unmet medical needs. Collectively, rare diseases are highly diverse. FDA is committed to helping sponsors of drugs for rare diseases create successful programs that address the particular challenges posed by each disease.

This guidance addresses the following important components of drug development:

- Adequate description and understanding of the disease's natural history
- Adequate understanding of the pathophysiology of the disease and the drug's proposed mechanism of action
- Nonclinical pharmacotoxicology considerations to support the proposed clinical investigation(s)
- Standard of evidence to establish safety and effectiveness
- Drug manufacturing considerations during drug development

Early consideration of these issues allows sponsors to efficiently and adequately address them during the course of drug development, from drug discovery to confirmatory efficacy and safety studies, and to have productive meetings with FDA.

This draft guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The draft guidance, when finalized, will represent the current thinking of FDA on common issues in drug development for rare diseases. It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations.

II. The Paperwork Reduction Act of 1995

This guidance refers to previously approved collections of information that are subject to review by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (44 U.S.C. 3501-3520). The collections of information in 21 CFR part 312 have been approved under OMB control number 0910-0014, and the collections of information in 21 CFR part 314 have been approved under OMB control number 0910-0001.

III. Comments

Interested persons may submit either electronic comments regarding this document to http://www.regulations.gov or written comments to the Division of Dockets Management (see ADDRESSES). It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at http://www.regulations.gov.

IV. Electronic Access

Persons with access to the Internet may obtain the document at http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm, http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/default.htm, or http://www.regulations.gov.

Dated: August 12, 2015.

Leslie Kux,

Associate Commissioner for Policy.

[FR Doc. 2015-20235 Filed: 8/14/2015 08:45 am; Publication Date: 8/17/2015]